

Sunset Public Hearing Questions for
Tennessee Rare Disease Advisory Council
Created by Section 71-7-101, *Tennessee Code Annotated*
(Sunset Termination June 2022)
CONTACT: Chair and Will Walker, Jonathan Singer

Enabling Statute, Purpose, and Rules and Regulations

1. Please provide a brief introduction to the advisory council including information about its purpose, statutory duties, staff, and administrative attachment.

The Tennessee Rare Disease Advisory Council's (TN-RDAC) mission is to improve the quality of life and to support care for Tennesseans affected by rare disease through collaboration, education, support and advocacy. Our council seeks to support policy development relevant to Tennesseans impacted by rare diseases, including advising TennCare's Pharmacy Advisory Committee and Drug Utilization Review Committee on pharmaceutical treatments. Additionally, we aim to serve as a resource for policymakers seeking to understand the overall impact of rare diseases on Tennesseans and identify strategies to improve healthcare for this population.

The TN-RDAC is administratively attached to the bureau of TennCare, but it has no staff. All duties are performed by the volunteer members of the council and by other volunteers recruited by council members. Lora Underwood, Director of Clinical Pharmacy Services for TennCare, is a member of the TN-RDAC and serves as a liaison to TennCare. Created through [SENATE BILL 2124/House Bill 2505](#) the TN-RDAC was established to "advise the TennCare prescription drug utilization review committee (DUR), the TennCare pharmacy advisory committee (PAC), and other public and private agencies in this state in providing services for persons diagnosed with rare diseases."

By statute, "the advisory council will advise the DUR and PAC in their review of products or medications for the treatment of rare and orphan diseases, and drugs or biological products within the emerging fields of personalized medicine and non-inheritable gene editing therapeutics. The advisory council will have no authority on any matter relating to TennCare, nor may it require the DUR or PAC to follow its recommendations. The DUR and PAC will consider information submitted to them by the advisory council on rare diseases and personalized medicine when making recommendations or determinations regarding:

(1) Beneficiary access to drugs and biological products for rare diseases, as defined in the federal Orphan Drug Act of 1983;

(2) Drugs and biological products that are approved by the United States food and drug administration; and

(3) The emerging fields of personalized medicine and non-inheritable gene-editing therapeutics.

The DUR and PAC will seek the input of the advisory council on rare diseases and personalized medicine to address topics for consultation, including, but not limited to:

(1) Rare diseases;

(2) The severity of rare diseases; and

(3) The demographics and the clinical description of patient populations.

This bill states that it does not require the advisory council, the DUR, or the PAC to consult with a person on any matter or requires those entities to meet with any specific expert or stakeholder.”

The TN-RDAC

(1) Will be administratively attached to the bureau of TennCare for the purpose of submitting meeting minutes compiled by the council secretary to the DUR and PAC; and

(2) Will advise other state entities in addition to the DUR and PAC.

2. Does the advisory council have rule promulgation authority? If yes, please cite the reference(s). If no, does the advisory council need rulemaking authority?

No, the advisory council does not have rule promulgation authority.

Advisory Council Organization

3. Provide a list of current advisory council members and explain how membership complies with Section 71-7-102, *Tennessee Code Annotated*.

By statute, the RDAC must include the following categories of members. Appointment lengths are staggered from 1-3 years in the first 3 years, and from thereafter are 3 years in length:

- One representative from TennCare
- Two representatives from academic research institutions
- Two physicians licensed and practicing in this state
- One geneticist or genetic counselor licensed and practicing in this state
- One registered nurse or advanced practice registered nurse practicing in the state
- Resident of this state who is 18 years of age or older and who has either 1)been diagnosed with a rare disease or 2)is a caregiver for a person who has been diagnosed with a rare disease.
- One representative of a rare disease patient organization operating in this state
- One appointee by the Lt. Governor
- One appointee by the Speaker of the House

Current members of the RDAC:

Chairperson: Scott Strome, MD, Executive Dean of the University of Tennessee College of Medicine and Vice Chancellor for Clinical Affairs at UT Health Science Center. Represents an academic research institution and is a physician licensed in the state.

Vice Chairperson: Terry Jo Bichell, RN, MPH, PhD, neuroscience researcher and Tennessee State Ambassador for the Rare Action Network of the National Organization for Rare Disorders (NORD); founder and director of COMBINEDBrain. (appointed

Secretary: Gillian Hooker, PhD ScM, CGC, is a genetic counselor and Chief Scientific Officer at Concert Genetics, an Adjunct Associate Professor at Vanderbilt University Medical Center and Immediate Past-President of the National Society of Genetic Counselors. General Members

J. Clay Callison, MD, Pulmonary Medicine, Vice President and Chief Medical Information Officer, UT Medical Center; director, Adult Cystic Fibrosis Center, UT Medical Center.

Eugene “Chip” Chambers, MD, Assistant Clinical Professor of Surgery, Vanderbilt University Medical Center; Founder and President, DADA2 Foundation, a patient advocacy group.

Reginald French, President and Chief Executive Officer, the Sickle Cell Foundation of Tennessee.

Rizwan Hamid, MD, PhD, Director, Division of Medical Genetics and Genomic Medicine, Department of Pediatrics, Vanderbilt University School of Medicine.

Suzanne Jackowski, PhD, Member, Recently retired from Department of Infectious Diseases, St. Jude Children’s Research Hospital

Megan Crow, BSN, flight nurse, Vanderbilt LifeFlight; Board Member and Secretary, Tennessee Hemophilia and Bleeding Disorders Foundation

Kim Stephens, DBA, a Communications, Diversity and Inclusion Consultant; President and Chief Executive Officer, Project Alive, a patient advocacy organization committed to finding and funding a cure for Hunter Syndrome

Lora Underwood, PharmD, Director of Clinical Pharmacy Services, Bureau of TennCare.

Abby Trotter, liaison Life Science Tennessee.

One representative from TennCare	Lora Underwood
Two representatives from academic research institutions	Scott Strome Rizwan Hamid J. Clay Callison Suzanne Jackowski
Two physicians licensed and practicing in this state	Scott Strome Rizwan Hamid Chip Chambers J. Clay Callison

One geneticist or genetic counselor licensed and practicing in this state	Rizwan Hamid Gillian Hooker
One registered nurse or advanced practice registered nurse practicing in the state	Megan Crow
Resident of this state who is 18 years of age or older and who has either 1)been diagnosed with a rare disease or 2)is a caregiver for a person who has been diagnosed with a rare disease.	Megan Crow Kim Stephens Terry Jo Bichell Chip Chambers
One representative of a rare disease patient organization operating in this state	Megan Crow Kim Stephens Terry Jo Bichell Reginald French Chip chambers
One appointee by the Lt. Governor	Kim Stephens
One appointee by the Speaker of the House	Rizwan Hamid

4. Are there any vacancies on the advisory council? If so, please indicate how long the position has been vacant and explain steps that have been taken to fill any vacancies.

There are no vacancies at this time.

5. How many times has the advisory council met since July 1, 2020? Please provide the date of advisory council meetings.

The council has met 11 times since July 1, 2020. The agenda and minutes of these meetings are available on the website: www.tnrdac.org

2020

- Oct 28
- Nov 25
- Dec 29

2021

- January 27
- Feb 24
- March 24
- April 28
- May 26

- June 23
- July 28
- August 25

6. How many members were present at each meeting? Please note meetings where the advisory council did not have a quorum.

There has not been a meeting where the council did not have a quorum. The average number of attendees has been 13.

Financial Information

7. What were the advisory council's revenues and expenditures since July 20, 2021? Please provide information about all funding sources.

The council does not have a source of revenue, nor does it have a budget for expenditures. The website was built and is maintained through the University of Tennessee.

8. Do advisory council members receive per diem or travel reimbursements? How much was paid to individual advisory council members since July 1, 2020?

No, the council does not reimburse travel. All meetings have been conducted on zoom.

9. Does the advisory council collect fees? If yes, provide relevant information about fees collected. Indicate whether fees were established through rule or through state law.

No, the council does not collect fees.

Sunshine Law, Public Meetings, and Conflict of Interest Policies

10. Is the advisory council subject to Sunshine law requirements (Section 8-44-101 et seq., *Tennessee Code Annotated*) for public notice of meetings, prompt and full recording of minutes, and public access to minutes? If so, what procedures does the advisory council have for informing the public of meetings and making minutes available to the public?

The meetings are publicly noticed on the website www.tnrdac.org. Agenda and minutes are placed on the website. Meetings are recorded and recordings also placed on the website.

11. Does the advisory council allow public comment at meetings? Is prior notice required for public comments? If public comment is not allowed, how does the advisory council obtain feedback from the public and those they regulate?

Yes the council allows public comment. There is currently no notice required.

12. Does the advisory council have policies to address potential conflict of interest by advisory council members, employees, or other state employees who work with the advisory council?

Upon initiation of the TN-RDAC, all members were asked to complete a declaration of relationships form to disclose any and all pertinent and potential conflicts of interest. Responses were reviewed by the TN-RDAC leadership. Additionally, members are asked to disclose relevant conflicts of interest as they arise during council discussions.

Advisory council Responsibilities

13. What steps has the advisory council taken to comply with Section 71-7-103, *Tennessee Code Annotated*, which requires the advisory council to advise certain state entities in their review of products or medications for the treatment of rare and orphan diseases, and drugs or biological products within the emerging fields of personalized medicine and non-inheritable gene editing therapeutics?

We have appointed member liaisons to the PAC and the DUR and ensure that a liaison is present at all meetings of the PAC and the DUR. Prior to quarterly PAC meetings, the agenda for the meetings is shared with members of the TN-RDAC, so that all can review to identify products or medications for the treatment of rare and orphan diseases. If such treatments are identified, comments are shared with the liaisons and a request will be made for an opportunity to submit comments.

Additionally, we have invested time during monthly TN-RDAC meetings to educate our members on the activities of the PAC and DUR so that we can most effectively serve in our advisory capacity. Representatives from the PAC and the DUR have presented background on their processes and procedures during RDAC meetings and provided their perspectives on how the TN-RDAC can best serve the PAC and DUR in areas pertaining to rare disease.

Specific areas of need are starting to be identified and strategies to collaborate on solutions are forthcoming.

Section 71-7-103 copied below:

71-7-103. Rare disease advisory council recommendations — Use of information — Input of advisory council.

(a)

(1) The Tennessee rare disease advisory council shall advise the DUR, the PAC, and other state entities in their review of products or medications for the treatment of rare and orphan diseases, and drugs or biological products within the emerging fields of personalized medicine and non-inheritable gene editing therapeutics.

(2)

(A) All advisory council recommendations must be presented in writing to members of the PAC and DUR and explained to members of the PAC and DUR by representatives of the advisory council during their public meetings.

(B) The advisory council has no authority on any matter relating to TennCare, nor may it require the DUR or PAC to follow its recommendations.

(b) The DUR and PAC shall consider information submitted to them by the advisory council on rare diseases and personalized medicine when making recommendations or determinations regarding:

- (1) Beneficiary access to drugs and biological products for rare diseases, as defined in the federal Orphan Drug Act of 1983 (21 U.S.C. §§ 360aa — 360ff-1);
 - (2) Drugs and biological products that are approved by the United States food and drug administration; and
 - (3) The emerging fields of personalized medicine and non-inheritable gene-editing therapeutics.
- (c) The DUR and PAC shall seek the input of the advisory council on rare diseases and personalized medicine to address topics for consultation under this chapter, including, but not limited to:
- (1) Rare diseases;
 - (2) The severity of rare diseases;
 - (3) The unmet medical needs associated with rare diseases;
 - (4) The impact of particular coverage, cost-sharing, tiering, utilization management, prior authorization, medication therapy management, or other medicaid policies on access to rare disease therapies;
 - (5) An assessment of the benefits and risks of therapies to treat rare diseases;
 - (6) The impact of coverage, cost-sharing, tiering, utilization management, prior authorization, medication therapy management, or other medicaid policies on patients' adherence to the treatment regimen prescribed or otherwise recommended by their physicians;
 - (7) Whether beneficiaries who need treatment from or a consultation with a rare disease specialist have adequate access and, if not, what factors are causing the limited access; and
 - (8) The demographics and the clinical description of patient populations.
- (d) Nothing in this chapter requires the advisory council, the DUR, or the PAC to consult with a person on any matter or requires those entities to meet with any specific expert or stakeholder.

Reports, Major Accomplishments, and Proposed Legislative Changes

14. What reports does the advisory council prepare concerning its activities, operations, and accomplishments? Who receives copies of these reports? Please provide a link to any such reports issued in the last two fiscal years.

The council plans to do an annual report, at the end of each calendar year.

15. What have been the advisory council's major accomplishments since July 1, 2020?

The council has held regular meetings since November 2020. Officers have been appointed and procedures established for meeting scheduling, agenda setting, forming liaison relationships and providing transparency into committee activities.

Our website, tnrdac.org, was launched in March 2021 and provides a valuable tool for connect with the community and making minutes and meeting recordings available.

Also in March 2021, we launched a state-wide survey of patients with rare diseases in the state to understand what resources exist and what obstacles exist. To date we have received 87 responses from across the state.

Each of our monthly meetings starts with a patient story, where we invite individuals with rare diseases to join and share their journeys with rare disease. These exchanges have provided a nuanced, detailed picture of the challenges faced by individuals with rare disease in the state

and have given rise to specific initiatives within the TN-RDAC which are aimed at promoting access to diagnostics and treatments for rare disease.

In the area of diagnostics, the council has discussed often long and costly diagnostic odysseys faced by those with rare diseases. A subgroup of the council has begun to outline a plan to connect institutions across the state to form a stronger diagnostic network to identify those with rare disease at earlier stages and provide support and referral guidance to healthcare providers across the state who may see patients with rare diseases. Such a project could also provide grounds for innovation and discovery research in rare diseases.

In the areas of treatment, we have discussed inefficiencies in care for rare diseases and specific areas where difficulties in access to treatment may lead to costly and unnecessary inpatient hospital stays. One specific area where such challenges arise is in the area of medical supplementation of individuals with rare metabolic diseases.

16. What, if any, challenges has the advisory council addressed since its creation?

The lack of a budget and administrative support is a challenge.

17. Please describe any items related to the advisory council that require legislative attention and your proposed legislative changes.

The statute establishing our council has provided an appropriate and effective guide in allowing the TN-RDAC to launch and begin operations.

Through our discussions, data collection and community exchanges, we have identified opportunities within TN for a more efficient, accurate and cost-effective diagnostic and referral process for rare diseases. To that end, we are developing a proposal for a novel and innovative program called the "Tennessee Rare and Undiagnosed Disease Network." Support from the legislation could advance this program and result in both improved care and cost savings for the state of TN.

18. Should the advisory council be continued? To what extent and in what ways would the absence of the advisory council affect the public welfare of the citizens of Tennessee?

The need for the RDAC in the State of TN is clear and the advisory council should be continued. More and more rare diseases are being diagnosed and treated every day, with targeted therapies in development. Our state needs to be ready to review and make policy on care for TN citizens with rare diseases.

19. Please identify the appropriate agency representative or representatives possessing substantial knowledge and understanding of the responses provided to the sunset review questions.

Dr. Scott Strome, Dr. Terry Jo Bichell, Dr. Gillian Hooker or Dr. Rizwan Hamid.

20. Please identify the appropriate agency representative or representatives who will respond to the questions at the scheduled sunset hearing.

Dr. Scott Strome, Dr. Terry Jo Bichell, Dr. Gillian Hooker or Dr. Rizwan Hamid.

21. Please provide the office address, telephone number, and email address of the agency representative or representatives who will respond to the questions at the scheduled sunset hearing.

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